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INTERNATIONAL PRELIMINARY EXAMINATION REPORT

(PCT Article 36 and Rule 70)

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| Applicant's or agent's file reference 10589-40-228 | | FOR FURTHER ACTION See Notification of Transmittal of International Preliminary Examination Report (Form PCT/PEPA/416) | |
| International application No. PCT/US03/19760 | International filing date (day/month/year) 23 June 2003 (23.06.2003) | Priority date (day/month/year) 23 June 2002 (23.06.2002) | |
| International Patent Classification (IPC) or national classification and IPC IPC(7): C12Q 1/68; C07H 21/02, 21/04 and US CL: 435/6; 536/23.1, 24/3 | | | |
| Applicant PTC THERAPEUTICS, INC. | | | |

1. This international preliminary examination report has been prepared by this International Preliminary Examining Authority and is transmitted to the applicant according to Article 36.
2. This REPORT consists of a total of 3 sheets, including this cover sheet.
- ☐ This report is also accompanied by ANNEXES, i.e., sheets of the description, claims and/or drawings which have been amended and are the basis for this report and/or sheets containing rectifications made before this Authority (see Rule 70.16 and Section 607 of the Administrative Instructions under the PCT).

These annexes consist of a total of ___ sheets.

3. This report contains indications relating to the following items:

- I ☒ Basis of the report
- II ☐ Priority
- III ☐ Non-establishment of report with regard to novelty, inventive step and industrial applicability
- IV ☐ Lack of unity of invention
- V ☒ Reasoned statement under Article 35(2) with regard to novelty, inventive step or industrial applicability; citations and explanations supporting such statement
- VI ☐ Certain documents cited
- VII ☐ Certain defects in the international application
- VIII ☐ Certain observations on the international application

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|---|--|
| Date of submission of the demand 16 January 2004 (16.01.2004) | Date of completion of this report 03 May 2005 (03.05.2005) |
| Name and mailing address of the IFEA/US Mail Stop PCT, Attn: IFEA/US Commissioner for Patents P.O. Box 1450 Alexandria, Virginia 22313-1450 Facsimile No. (703) 305-3230 | Authorized officer Ethan Whisenant, Ph.D. Telephone No. (571) 272-1600 |

Form PCT/PEPA/409 (cover sheet) (July 1998)

Calvin Bell-Hank
Ethan Whisenant
PRIMARY EXAMINER

INTERNATIONAL PRELIMINARY EXAMINATION REPORT

International application No.

PCT/US03/19760

I. Basis of the report

1. With regard to the elements of the international application:*

- ☒ the international application as originally filed.
- ☒ the description:
pages 1-241 _____, as originally filed
pages NONE _____, filed with the demand
pages NONE _____, filed with the letter of _____
- ☒ the claims:
pages 242-246 _____, as originally filed
pages NONE _____, as amended (together with any statement) under Article 19
pages NONE _____, filed with the demand
pages NONE _____, filed with the letter of _____
- ☒ the drawings:
pages 1-11 _____, as originally filed
pages NONE _____, filed with the demand
pages NONE _____, filed with the letter of _____
- ☐ the sequence listing part of the description:
pages NONE _____, as originally filed
pages NONE _____, filed with the demand
pages NONE _____, filed with the letter of _____

2. With regard to the language, all the elements marked above were available or furnished to this Authority in the language in which the international application was filed, unless otherwise indicated under this item.

These elements were available or furnished to this Authority in the following language _____ which is:

- ☐ the language of a translation furnished for the purposes of international search (under Rule 23.1(b)).
- ☐ the language of publication of the international application (under Rule 48.3(b)).
- ☐ the language of the translation furnished for the purposes of international preliminary examination (under Rules 55.2 and/or 55.3).

3. With regard to any nucleotide and/or amino acid sequence disclosed in the international application, the international preliminary examination was carried out on the basis of the sequence listing:

- ☐ contained in the international application in printed form.
- ☐ filed together with the international application in computer readable form.
- ☐ furnished subsequently to this Authority in written form.
- ☐ furnished subsequently to this Authority in computer readable form.
- ☐ The statement that the subsequently furnished written sequence listing does not go beyond the disclosure in the international application as filed has been furnished.
- ☐ The statement that the information recorded in computer readable form is identical to the written sequence listing has been furnished.

4. The amendments have resulted in the cancellation of:

- ☐ the description, pages NONE
- ☐ the claims, Nos. NONE
- ☐ the drawings, sheets/fig NONE

5. ☐ This report has been established as if (some of) the amendments had not been made, since they have been considered to go beyond the disclosure as filed, as indicated in the Supplemental Box (Rule 70.2(c)).**

* Replacement sheets which have been furnished to the receiving Office in response to an invitation under Article 14 are referred to in this report as "originally filed" and are not annexed to this report since they do not contain amendments (Rules 70.15 and 70.17).

** Any replacement sheet containing such amendments must be referred to under item 1 and annexed to this report.

INTERNATIONAL PRELIMINARY EXAMINATION REPORT

International application No.
PCT/US83/19760**V. Reasoned statement under Rule 66.2(a)(ii) with regard to novelty, inventive step or industrial applicability; citations and explanations supporting such statement****1. STATEMENT**

| | | |
|-------------------------------|--------------------|------------|
| Novelty (N) | Claims <u>1-19</u> | <u>YES</u> |
| | Claims <u>NONE</u> | <u>NO</u> |
| Inventive Step (IS) | Claims <u>1-19</u> | <u>YES</u> |
| | Claims <u>NONE</u> | <u>NO</u> |
| Industrial Applicability (IA) | Claims <u>1-19</u> | <u>YES</u> |
| | Claims <u>NONE</u> | <u>NO</u> |

2. CITATIONS AND EXPLANATIONS

1. Claim(s) 1-19 meet the criteria set out in PCT Article 33(2)-(3), because the prior art does not teach or fairly suggest a method of identifying a compound that modulates premature translation termination or nonsense-mediated mRNA decay comprising all of the limitations of Claims 1-5. In addition, the prior art does not teach or fairly suggest a method of identifying a compound to be tested for its ability to prevent or treat a disease characterized by or associated with the presence of a premature stop codon in a gene comprising all of the limitations of Claims 9-10.

2. Claim(s) 1-19 meet the criteria set out in PCT Article 33(4), and thus have industrial applicability because the subject matter claimed can be made or used in industry.

----- NEW CITATIONS -----